(12) INTERNATIONAL LICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date 12 February 2004 (12.02.2004)

PCT

(10) International Publication Number WO 2004/013280 A2

(51) International Patent Classification7:

C12N

(21) International Application Number:

PCT/US2003/016887

(22) International Filing Date:

26 May 2003 (26.05.2003)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

10/212,322 5 August 2002 (05.08.2002) US 10/322,086 17 December 2002 (17.12.2002) US 10/430,351 5 May 2003 (05.05.2003) US

(63) Related by continuation (CON) or continuation-in-part (CIP) to earlier application:

US Filed on 10/430,351 (CIP) 5 May 2003 (05.05.2003)

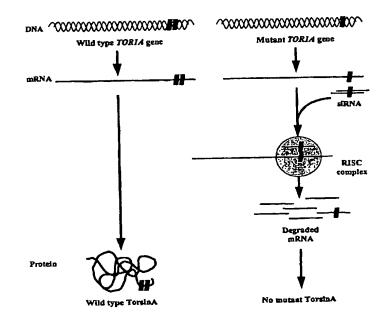
(71) Applicant (for all designated States except US): UNIVER-SITY OF IOWA RESEARCH FOUNDATION [US/US]; 100 Oakdale Campus, 214 Technology Information Center, Iowa City, IA 52242-5000 (US).

(72) Inventors; and

- (75) Inventors/Applicants (for US only): DAVIDSON, Beverly, L. [US/US]; 3640 Johnson Way N.E., North Liberty, IA 52242 (US). GONZALEZ-ALEGRE, Pedro [US/US]; Department of Neurology, University of Iowa College of Medicine, 2007 RCP, Iowa City, IA 52242 (US). MILLER, Victor [US/US]; Department of Neurology, University of Iowa College of Medecine, 2007 RCP, Iowa City, IA 52242 (US). PAULSON, Henry [US/US]; Department of Neurology, University of Iowa College of Medicine, 2007 RCP, Iowa City, IA 52242 (US). HARPER, Scott [US/US]; Department of Neurology, University of Iowa College of Medicine, 2007 RCP, Iowa City, IA 52242 (US).
- (74) Agents: STEFFEY, Charles, E. et al.; Schwegman, Lundberg, Woessner & Kluth, P.O. Box 2938, Minniapolis, MN 55402 (US).
- (81) Designated States (national): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PH, PL, PT, RO, RU, SC, SD,

[Continued on next page]

(54) Title: ALLELE-SPECIFIC siRNA-MEDIATED GENE SILENCING



(57) Abstract: The present invention is directed to small interfering RNA molecules (siRNA) targeted against an allele of interest, and methods of using these siRNA molecules.